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## RFA 07-04: CIRM DISEASE TEAM PLANNING AWARD

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### I. PURPOSE

Pluripotent stem cells have the potential to play a role in the development of treatments and diagnostics for chronic disease and serious injury. To date only a handful of stem cell-based therapies have progressed into clinical testing and practice. In part, this is because stem cell research is an emerging field. The purpose of the CIRM Disease Team Initiative is to explore a new method of integrating and organizing the highest quality basic, translational and clinical stem cell research with the specific aim of producing a therapy or diagnostic for a particular disease or serious injury. This approach allows the use of innovative research models such as requiring active team management and emphasizing defined milestones to better support and to accelerate research that is poised for the development of stem cell-based treatments. The Disease Team Initiative has three parts: a Workshop (held July, 2007), Planning Awards (which are the subject of this Request for Applications (RFA 07-04)), and the Disease Team Research Awards (which will be the subject of a subsequent RFA). This Disease Team Planning Award (RFA 07-04) will support up to six months of planning and proposal development for the Disease Team Research Awards.

### II. PROGRAM BACKGROUND AND OBJECTIVES

#### Background

The California Institute of Regenerative Medicine (CIRM) is charged with furthering the development of therapies, cures and diagnostics based on human stem cell research in California. To fulfill this ambitious mission, the CIRM developed a Scientific Strategic Plan in 2006 that defined the specific goals of the Institute and established a detailed blueprint for achieving these goals. Several funding programs targeting different aspects of biomedical research were proposed, among them the Disease Team Initiative to support teams composed of basic, translational and clinical scientists working synergistically to develop therapies and diagnostics for specific disease targets.

The Disease Team Initiative encourages novel team research models requiring active team management and emphasizing defined milestones in an effort to jump-start the development of therapies based on stem cells. The Disease Team Initiative complements other CIRM programs that are already in place (e.g. Comprehensive Research and SEED grants, the Training Program, New Faculty Awards) or that are planned to focus on specific stages in the pathway to stem cell-based therapies and diagnostics, such as the initiatives for Translational Research, Preclinical Product Development, and Clinical Investigation.

Given the novelty of the team-based research approach, CIRM divided the Disease Team Initiative into three parts: a Workshop, Planning Award, and Disease Team Research Award.

### **Workshop:**

To evaluate approaches for team-based research, CIRM conducted a Workshop in July 2007 inviting representatives from academia, industry, disease-focused foundations, and federal funding and regulatory agencies to explore the scope, resources, management and funding of effective teams, and to evaluate the strengths and weaknesses of various approaches to team-based research. Three main findings emerged from the Workshop (posted at <http://www.cirm.ca.gov/pub/default.asp> "Report of the Disease Team Workshop"):

1. Translational research does indeed benefit from team-based research, which encourages early consultation and cooperation among researchers of diverse skills and expertise.
2. Strong scientific leadership and project management are essential for team recruitment, motivation, and success.
3. Active management and oversight provided by the funding organization can maintain focus and increase the rate of successful translation to the clinic.

A key objective of a Disease Team Research Award will be to produce an approvable regulatory filing enabling clinical testing within four years after award. Therefore, a scientifically mature concept for translating basic and preclinical findings into clinical testing is critical. Robust Disease Team Research Award proposals will identify and describe many of the elements below:

- A scientifically mature opportunity that meets an unmet medical need, in which the use of human stem cells can offer an advantage over other approaches
- A path for translation of basic and preclinical findings to stem cell potential therapies within a specific disease or injury, including identification of and proposed solutions to current gaps and roadblocks
- Required expertise, resources, services and technologies to move the research forward and to overcome gaps and obstacles
- A method of integrating multidisciplinary team members
- A leadership philosophy and plan that motivates and sustains teams
- A project management plan that will focus on moving potential therapies into clinical testing
- Anticipated regulatory hurdles, and strategies by which to overcome these hurdles

### **Planning Award (Objectives):**

Because key elements of the disease-team research approach differ from traditional research conducted by individual investigators and require a high level of coordination, CIRM recognized that the Disease Team Initiative could benefit from a planning process. The objective of the Planning Awards (RFA 07-04) is to lay the foundation and prepare the team to write an application for a subsequent Disease Team Research Award. Planning Awards are intended to enable a Principal Investigator to recruit a team, and to enlist that team to plan the content, management, and administration of a team-based

research project. The research award requirements will be described in a subsequent RFA.

This planning process is intended to involve all significant Disease Team participants in addressing the objectives of the Disease Team Research. Teams might include members with the following expertise, either as full time or advisory members: basic research including but not limited to stem cell biology and immunology, animal modeling, specialization in treatment of a particular disease, transplantation, project management, pharmacology, toxicology, process development, quality control and assurance, biostatistics, regulation of biomedical products, and the conduct of clinical trials.

Among the key elements of Disease Team planning is discussion and development of:

- A scientifically mature opportunity, with strong preclinical proof-of-concept, to use stem cells in the development of a therapy or diagnostic; and the significance of the work
- A route to advance the potential therapy toward clinical testing, including:
  - A clear, step by step project plan, complete with milestones and deliverables
  - A justification for a team approach
- A description of the organization, management and plan for sustaining the Disease Team
- Plans for how the team will approach the anticipated regulatory hurdles

Planning Award applications must identify a disease target or serious injury target for which there is strong proof-of-concept, and describe a detailed and thoughtful planning process. This planning process should include identification and preliminary description of: 1) path for translation of basic and preclinical findings to stem cell potential therapies within a specific disease or injury, including identification of and proposed solutions to current gaps and roadblocks; 2) the expertise, resources, services and technologies to move the research forward and to overcome these gaps and obstacles; and 3) justification for why the proposed planning approach will be effective.

#### **Research Award:**

CIRM anticipates a call for applications for the multi-year Disease Team Research Award (the subject of a future RFA) following this planning cycle. CIRM believes that supporting the planning process will strengthen the pool of applications for subsequent Disease Team Research Awards, by allowing the teams to consider key strategic and operational issues faced when assembling a multidisciplinary team. Receipt of a Planning Award, however, will not be a prerequisite for a Disease Team Research Award. Upon completion of a Planning Award, recipients will be required to submit a Final Progress Report to CIRM.

### **III. AWARD INFORMATION**

The ICOC has approved total funding of \$1.1 million for the Disease Team Planning Awards. Each Planning Award will provide one-time only support for planning, organization of teams and initial development of a Disease Team Research Award

proposal. CIRM intends to provide project costs of up to \$50,000. Indirect costs will not exceed 10% of the total direct costs, with total costs (direct and indirect) not to exceed \$55,000 for each award. CIRM intends to support up to 20 awards for up to six months of effort.

CIRM intends to support projects targeting a broad range of diseases and injuries. The Disease Team Initiative will consider multiple roles for human stem cells in the development of therapies, including but not limited to: transplantation and integration, mobilizing endogenous cells, modifying the immune system, acting as delivery vehicles, and serving as disease models for drug screening and development (in cases in which no clinically relevant disease model exists to date). CIRM will support research using the full spectrum of stem cell types and experimental approaches, including human embryonic stem cells, induced pluripotent stem cells (iPS cells) as well as stem cells from adults, cord blood and other sources. Preference will be given to approaches that are not fundable by the federal government.

#### **IV. ELIGIBILITY INFORMATION**

Applications will only be accepted from Principal Investigators (PIs) that: 1) have been officially nominated on a Candidate Nomination Form (CNF, see RFA section VI A) by their home institution; and 2) have submitted a Letter of Intent (LOI, see RFA section VI B) that was accepted by CIRM.

##### **Institutional Eligibility**

This RFA is open to all academic and non-profit research institutions in California, as well as to for-profit organizations in California. Each academic or non-profit institution may submit up to four applications, each of which must address a different disease or serious injury. Each for-profit organization may submit one application.

Non-profit means either: (1) a governmental entity of the state of California; or (2) a legal entity that is tax exempt under Internal Revenue Code section 501(c)(3) and California Revenue and Taxation Code section 23701d.

For-profit means: an organization, institution, corporation, or other legal entity that is organized or operated for the profit or financial benefit of its shareholders or other owners. Such organizations also are referred to as “commercial organizations”.

##### **Principal Investigator (PI) Eligibility**

The PI is the individual who will be responsible for team assembly and organization, and development of the team’s proposal for a Disease Team Research Award. The PI must be a full-time employee (80 percent time) of the applicant institution at the time of submission of the application, and for the duration of the Planning Award period. No substitutions for the PI will be allowed for the duration of the Planning Award.

A single PI may submit only one application under this RFA. Candidates must have received an MD, PhD or equivalent degree. PIs must commit a minimum of 5 percent effort exclusively to the Disease Team planning proposed in their application.

## V. REVIEW CRITERIA

Applications will be evaluated in three areas: the Concept - Rationale, Maturity and Significance; the Principal Investigator; and the Planning Approach.

**Concept - Rationale, Maturity, and Significance:** Compelling data support the feasibility of the proposed concept, the underlying scientific hypothesis and approach. The PI has identified a plausible target(s), and a role for human stem cells to be explored during the planning process. The scientific concept is sufficiently mature, such that there is reasonable expectation for clinical studies within 5 years. The proposed research concept that will be investigated and developed during the planning process addresses a critical problem and/or an unmet medical need in the named disease or injury in which the use of human stem cells can offer an advantage over other approaches.

**Principal Investigator:** The PI is an acknowledged thought-leader and expert in the proposed field of study. The PI is appropriately trained and well suited to carry out this planning process (assemble the team, and lead the development of the research plan). The PI has a track record in the field, and the potential to successfully translate basic and preclinical findings into clinical studies.

**Planning Approach:** The proposed planning process is detailed and thoughtful, and includes personnel with the necessary expertise to ensure that the process is thorough and runs smoothly. The PI will foster coordination and collaboration among members of the planning team. The PI provides evidence that the planning process will provide the potential Disease Team with vision and clarity that would not otherwise be possible.

## VI. APPLICATION PROCEDURE

Applicant institutions and candidates must follow these instructions for submitting a Candidate Nomination Form, Letter of Intent, and Application for the CIRM Disease Team Planning Awards. Applications will only be accepted from PIs that: 1) have been officially nominated on a Candidate Nomination Form (CNF) by their home institution; and 2) have submitted a Letter of Intent (LOI) that was accepted by CIRM.

### A. Candidate Nomination Form (CNF)

Applicant institutions must submit to CIRM a single Candidate Nomination Form (CNF) using the CNF template provided at <http://www.cirm.ca.gov/grants/default.asp>. The CNF must list the name, degree, and employment title of each of the PI(s) the institution wishes to nominate for these awards, and the disease or serious injury that will be the focus of the planning grant. CIRM will accept only one CNF from each institution; this form must be signed by an institutional official authorized to nominate candidates on behalf of the entire institution. The signed original CNF must be received by CIRM no later than **5:00pm (PST) on December 20, 2007. No exceptions will be made.**

Mail the signed original CNF to:

Disease Team Planning Award Candidate Nomination Form  
California Institute for Regenerative Medicine  
210 King Street  
San Francisco, CA 94107

## **B. Letter of Intent**

Candidates for the award must submit a Letter of Intent (LOI) using the LOI template provided at <http://www.cirm.ca.gov/grants/default.asp>. The letter should describe concisely the disease target or serious injury target, and summarize the concept for which planning activities will occur. Completed LOIs should be sent as an email attachment to [PlanningAward\\_loi@cirm.ca.gov](mailto:PlanningAward_loi@cirm.ca.gov), and must be received by CIRM no later than **5:00PM (PST) on December 20, 2007. No exceptions will be made.** Letters of intent are non-binding, but applications will not be accepted if an LOI has not been received by CIRM by the stated deadline.

## **C. Application Instructions**

The application for CIRM Disease Team Planning Award consists of three parts:

Part A: Application Information Form (Adobe PDF template provided at <http://www.cirm.ca.gov/grants/default.asp>.) Part A includes: Abstract, Public Abstract, Statement of Benefit to California and Budget (section numbers 1, 2, 3 and 8 below).

Part B: Disease Team Planning Award Research Proposal (MS Word template provided at <http://www.cirm.ca.gov/grants/default.asp>.) Part B includes: Concept - Rationale, Maturity and Significance; Principal Investigator; Planning Approach; and References (section numbers 4,5,6 and 7 below).

Part C: Biographical Sketch for Principal Investigator (MS Word template provided at <http://www.cirm.ca.gov/grants/default.asp>.)

All three parts of the application for a CIRM Disease Team Planning Award must be submitted together and received by CIRM no later than **5:00PM (PST) on January 31, 2008. No exceptions will be made.** Candidates must use the appropriate CIRM templates to complete Parts A, B and C. These templates will be available on the CIRM website (<http://www.cirm.ca.gov/grants/default.asp>) by December 11, 2007.

The application for Disease Team Planning Awards includes the following sections:

- 1. Abstract (up to 3000 characters in Part A)*

State the goals of the proposal; summarize the overall plans of the proposed concept and how it will meet the stated objectives of the RFA. Describe the rationale for choosing the specific disease target or serious injury target. Summarize the approach that will be undertaken to assemble a team and prepare a research proposal for the Disease Team Research Award.

2. *Public Abstract (up to 3000 characters in Part A)*  
Briefly describe in lay language the proposed concept and how it will, directly or indirectly, contribute to the development of therapies or diagnostics. This Public Abstract will become public information; therefore, do not include proprietary or confidential information or information that could identify the candidate and applicant institution.
3. *Statement of Benefit to California (up to 3000 characters in Part A)*  
Describe in a few sentences how the proposed research will benefit the state of California and its citizens. This Statement of Benefit will become public information; therefore, do not include proprietary or confidential information or information that could identify the candidate and applicant institution.
4. *Concept - Rationale, Maturity and Significance (up to 2 pages in Part B)*  
Describe the disease or serious injury target, and summarize the concept around which planning activities will be organized. Summarize the evidence indicating that a disease team research approach could lead to clinical studies. Evaluate existing knowledge and specifically identify the gaps that the planning process is intended to explore. State how the proposed concept meets CIRM's primary goal for the Disease Team Initiative, advancing a stem cell-derived therapy or diagnostic toward the clinic.
5. *Principal Investigator and Feasibility (up to 1 page in Part B)*  
Summarize the track record and/or the potential the PI has in successfully translating basic and preclinical findings into clinical studies. Describe any past experience with assembling a multidisciplinary team, and carrying out planning efforts and team proposal development.
6. *Planning Approach (up to 1 page in Part B)*  
Describe the process to assemble the team, to identify potential projects, and to develop a disease team proposal. Specifically address how the PI will identify a multidisciplinary team that will collaboratively develop a research plan, and implement active management. Describe how potential team members will coordinate and collaborate in this process.
7. *References (up to 1 page in Part B)*  
List all references used in the body of the proposal.
8. *Budget (included in Part A)*  
Provide all budget information requested in the budget section of in the application form. All allowable costs for research grants are detailed in the CIRM Grants Administration Policy for Academic and Non-profit Institutions (Non-Profit GAP, [http://www.cirm.ca.gov/reg/pdf/reg100500\\_policy.pdf](http://www.cirm.ca.gov/reg/pdf/reg100500_policy.pdf)), and in the Interim CIRM Grants Administration Policy for For-Profit Organizations (For-Profit GAP, <http://www.cirm.ca.gov/policy/policy.asp>) which will be available by December 19, 2007. Under this RFA, allowable costs include the following:

- **Salary for Principal Investigator**

Salary support may be requested for the Principal Investigator. Salaries for PIs are based on percent of full-time effort commensurate with the established salary structure of the applicant institution. Salaries are only paid for PIs whose primary place of employment is located in California. The total salary requested by the PI must be based on an annualized, 12-month staff appointment, and no more than 20% of the total award can be used for PI salary costs. Administrative support salaries are expected to be covered exclusively by allowed Indirect Costs.

- **Consultants**

Consultant expenses may be requested to pay the costs of engaging seminar speakers or outside experts to provide professional advice or services to assist in planning. Potential collaborators and personnel who are located and employed outside the PI's home institution may request compensation as a consultant in this section. CIRM funds cannot be used for salary support of individuals working outside of California.

- **Travel and Communications**

Travel expenses may be requested to pay for travel to attend planning meetings. Travel costs associated with bringing together potential collaborators to work on the Disease Team plan are allowable. Further information about allowable travel costs can be found in the CIRM Grants Administration Policy for Academic and Non-profit Institutions (Non-Profit GAP, [http://www.cirm.ca.gov/reg/pdf/reg100500\\_policy.pdf](http://www.cirm.ca.gov/reg/pdf/reg100500_policy.pdf)) and in the Interim CIRM Grants Administration Policy for For-Profit Organizations (For-Profit GAP, <http://www.cirm.ca.gov/policy/policy.asp>) which will be available by December 19, 2007. Communications expenses may be requested to pay for remote conferencing, audio-visual equipment rental, costs of developing meeting materials and if required, conference room leasing.

- **Indirect Costs**

Program administration costs will be covered by indirect costs, which will be limited to no more than 10 percent of allowable direct funding costs awarded by CIRM. No facilities costs will be provided.

## **VII. SUBMITTING AN APPLICATION**

Applications will only be accepted from PIs who 1) have been officially nominated on a CNF from their home institution and 2) have submitted a Letter of Intent (LOI) that was accepted by CIRM.

The application for CIRM Disease Team Planning Awards consists of three parts:

Part A: Application Information Form (Adobe PDF template provided at <http://www.cirm.ca.gov/grants/default.asp>.)

Part B: Disease Team Planning Award Research Proposal (MS Word template provided at <http://www.cirm.ca.gov/grants/default.asp>.)

Part C: Biographical Sketches for Principal Investigator (MS Word template provided at <http://www.cirm.ca.gov/grants/default.asp>.)

All three parts of the application for CIRM Disease Team Planning Awards must be submitted together and received by CIRM no later than **5:00PM (PST) on January 31, 2008. No exceptions will be made.** Candidates must use the CIRM templates to complete Parts A, B and C. These templates will be available on the CIRM website by December 11, 2007. Send electronic copies of all three parts of the application as attachments in a single email to [PlanningAwards@cirm.ca.gov](mailto:PlanningAwards@cirm.ca.gov) no later than **5:00PM (PST) on January 31, 2008. No exceptions will be made.** In addition to the electronic submittal, candidates must submit an original copy of the application signed by both the PI and the institution's AOO, plus 5 copies (preferably double-sided) of the full application to:

Disease Team Planning Award Application  
California Institute for Regenerative Medicine  
210 King Street  
San Francisco, CA 94107

The original application plus the five copies must be received by CIRM no later than **5:00PM (PST) on January 31, 2008. No exceptions will be made.**

#### **VIII. SCHEDULE OF RECEIPT AND ANTICIPATED REVIEW**

<b>Receipt of Candidate Nomination Forms and Letters of Intent:</b>	<b>5:00PM (PST) on December 20, 2007</b>
<b>Receipt of Applications:</b>	<b>5:00PM (PST) on January 31, 2008</b>
<b>Review of Applications by Grants Working Group (GWG):</b>	<b>April, 2008</b>
<b>Review and Approval by ICOC:</b>	<b>June, 2008</b>
<b>Earliest Funding of Awards:</b>	<b>July, 2008</b>

#### **IX. REVIEW AND AWARD PROCESS**

CIRM Disease Team Planning Award applications will be reviewed by the CIRM Scientific and Medical Research Funding Working Group (the Grants Working Group, or GWG). The GWG consists of fifteen basic and clinical scientists from institutions outside California, seven patient advocates who are members of the Independent Citizen's Oversight Committee (ICOC), and the Chair of the ICOC. The membership of the GWG can be found at <http://www.cirm.ca.gov/workgroups/pdf/GrtWkgGpMbr.pdf>. The ICOC was established by the California Stem Cell Research and Cures Act (Proposition 71) to oversee CIRM and makes all final funding decisions. The composition of the ICOC can be viewed at <http://www.cirm.ca.gov/faq/pdf/Members.pdf>.

Fifteen scientists on the GWG will review the applications and score their scientific merit. For Disease Team Planning Award applications, particular emphasis will be placed on

the scientific maturity of the concept, the qualifications of the Principal Investigator, and the planning approach.

The full membership of the GWG will then review the entire portfolio of applications, taking into consideration the following criteria:

- Appropriate balance between feasibility and innovation.
- Where relevant, the appropriate balance and range of diseases addressed.
- Other considerations from the perspective of patient advocates.

The GWG's final recommendations for funding will then be forwarded to the ICOC, which will make all final funding decisions.

## **X. CONTACTS**

### For review information:

Bettina Steffen, M.D.  
Scientific Officer  
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210 King Street  
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Email: [gsambrano@cirrn.ca.gov](mailto:gsambrano@cirrn.ca.gov)  
Phone: (415) 396-9103  
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### For information about electronic forms:

Ed Dorrington  
Director of Grants Management Systems  
California Institute for Regenerative Medicine  
210 King Street  
San Francisco, CA 94107  
Email: [edorrington@cirrn.ca.gov](mailto:edorrington@cirrn.ca.gov)  
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For programmatic information:

Patricia Olson, Ph.D.  
Interim Director of Scientific Activities  
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210 King Street  
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Phone: (415) 396-9116  
FAX: (415) 396-9141

## **XI. OTHER REQUIREMENTS**

### **A. CIRM Grants Administration Policy**

CIRM's Grants Administration Policy for Academic and Non-profit Institutions (Non-Profit GAP) and for For-Profit entities (For-Profit GAP) serve as the standard terms and conditions of grant awards issued by CIRM. All research conducted under this award must comply with the stated policy, which can be found on the CIRM website: (Non-Profit GAP, [http://www.cirm.ca.gov/reg/pdf/reg100500\\_policy.pdf](http://www.cirm.ca.gov/reg/pdf/reg100500_policy.pdf)) or (For-Profit GAP, <http://www.cirm.ca.gov/policy/policy.asp>). CIRM intends to make the Interim For-Profit GAP available by December 19, 2007, subject to approval by the ICOC.

### **B. Evaluation of the Program**

In fulfilling our commitment to the State of California, CIRM may request information essential to an assessment of the effectiveness of this program. Accordingly, recipients are hereby notified that they may be contacted after the completion of this award for periodic updates on various aspects of their employment history, publications, support from research grants or contracts, honors and awards, professional activities, and other information helpful in evaluating the impact of the program. CIRM also retains the right audit all expenditures of CIRM funds.

### **C. Intellectual Property Policy**

CIRM's Intellectual Property policies will not apply to the Planning Awards, but will apply to any subsequent Disease Team Research Awards. CIRM has adopted regulations governing intellectual property resulting from CIRM-funded research at non-profit institutions ([http://www.cirm.ca.gov/reg/pdf/IP\\_Regs\\_100300.pdf](http://www.cirm.ca.gov/reg/pdf/IP_Regs_100300.pdf)). For-profit organizations will be regulated by an intellectual property policy as adopted by the ICOC prior to final approval of applications under the Disease Team Research Awards RFA (<http://www.cirm.ca.gov/faq/pdf/ForProfitOrg.pdf>).